Fact Sheet: Prochymal™ Therapy as a Medical Countermeasure for Acute Radiation Syndrome

Introduction

In response to the growing threat of nuclear and radiological terrorism, effective medical countermeasures must be developed to address Acute Radiation Syndrome (ARS) or "radiation sickness." Prochymal is being developed as a treatment for the complications of ARS that are not addressed by currently available medical countermeasures.

ARS

ARS occurs following exposure to high levels of radiation, inducing damage to the gastrointestinal (GI) tract and skin, and a reduction in the body's ability to produce blood forming cells and platelets.

Drug Description

Prochymal is a formulation of adult stem cells (mesenchymal stem cells, or "MSCs"), derived from the bone marrow of healthy adult donors. Prochymal does not contain embryonic cells or any constituent derived from embryonic or fetal sources.

Efficacy Data

Prochymal has shown improved outcomes in patients exposed to high levels of radiation and addresses clinical manifestations of ARS:

- **Survival** – Improved survival observed following administration of Prochymal in humans exposed to high levels of total body irradiation (9-14 Gy).
- **Gastrointestinal (GI) Repair** – Human clinical trials demonstrate a direct reparative effect on the GI tract.
- **Skin Repair** – Rapid healing and improved outcomes demonstrated in clinical studies.
- **Immune System and Blood Formation Recovery** – Improved recovery of blood cell counts and engraftment observed in clinical trials.

Safety and Regulatory Status

Prochymal has demonstrated an excellent safety profile in well controlled human trials and has advanced to the final stage of testing before approval:

- Approvable therapy in advanced stage of development. Currently in Phase III with FDA Fast Track designation for diseases with clinical features similar to ARS.
- Safety profile includes 53 patient placebo-controlled clinical trial of intravenous administration of Prochymal that met all safety endpoints.
- Safety evaluation includes thorough toxicology program developed in coordination with FDA.

Practical Advantages

- Prochymal therapy can be administered post-exposure, including at the onset of symptoms. There is no pre-defined treatment window.
- Prochymal can be administered to patients in cases where prophylactic measures have not yielded satisfactory clinical results.
- Osiris has established the ability to mass produce Prochymal in compliance with FDA and EU GMP regulations. The strong stability profile permits long term product storage.
- Osiris and Genzyme have partnered for the development of Prochymal for ARS to ensure that the necessary resources are available to the program.

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